17th April 2023

Dear Dr Kewalramani

We are writing to you as cystic fibrosis (CF) patients, family members and advocates that represent thousands of families from more than 60 countries around the world, many without access to your life-saving medicines; from Brazil, India, Lebanon, Lithuania, South Africa and Ukraine, to name a few. In these countries families are in a torturous situation, watching lives being transformed in the richest nations while their precious children and loved-ones continue to suffer and die.

In 2022 alone, you made $9 billion from cystic fibrosis product revenues and total CF revenues of $38.5 billion in the 11 years since your first CF drug was approved. You personally took home compensation worth $15.9 million in 2022. You have made exorbitant profits and personal gain off the back of our community and it is now imperative that you recognise your responsibility to CF patients around the world. You have a monopoly in CF modulator production but you have a moral obligation to use that power, not just to make profit but to ensure that everyone has access to these lifesaving medicines.

Our petition calling for global equitable access for all reached 51,000 signatures this week and continues to grow, demonstrating the level of support that we have – the CF community and wider public are speaking out and we want you to listen and take immediate action to end this intolerable suffering.

In February this year we launched the Right to Breathe campaign, with patients from South Africa, Brazil, Ukraine and India pushing for legal intervention and flexibilities to be used so that cystic fibrosis patients in these countries can gain access to Trikafta. Here are details of what is happening in each country:

South Africa
There are approximately 550 diagnosed cases of CF in South Africa, and likely many more undiagnosed. There is no access to any of your cystic fibrosis transmembrane conductance regulator (CFTR) modulators. And despite patenting your products in South Africa, you have not engaged with the government or health department in any meaningful way to try to ensure access. Your prices are completely unaffordable for almost all South Africans - a citizen earning the average annual wage in SA (approximately $24,000) would need to dedicate their entire income over 13 and a half years to buy just one year's supply of elexacaftor/tezacaftor/ivacaftor (Trikafta).

Cheri Nel, a South African CF patient, filed an affidavit with the South African High Court, asking for a declaration that your conduct is an abuse of your patent rights in terms of section 56 of the Patents Act. She is seeking a compulsory licence that will end your monopoly protection in South Africa, and allow for the supply of a more affordable generic version of the medicine.

We know that you have given notice of your intention to challenge the compulsory licence request, and in doing so, you are harming patients' lives. We ask you withdraw your challenge to the request, significantly drop your price in South Africa and begin immediate engagement with the South African government to ensure access or facilitate generic supply through the issuance of voluntary licences.

Brazil

There are approximately 6600 diagnosed cases of CF in Brazil, only a handful of these patients have access to CFTR modulators. In a preliminary ruling the Brazilian health system has just announced that the price of Trikafta is too high and will not be made available to Brazilians through the public health system.

The Brazilian CF Association on behalf of members of the CF community and a coalition of health and human rights organisations in Brazil submitted a formal written request to the Health Ministry on the 6th February 2023, requesting a CL for CFTRs. Given the recent ruling this request becomes even more urgent. For the sake of Brazilian patients we urge you to drop your patent claims in Brazil, dramatically reduce your prices or help facilitate generic supply of CFTRs as a matter of urgency.

India

There are approximately 600 diagnosed cases of CF in India, but it is estimated that there are approximately 37,000 undiagnosed patients. Recent research suggests there could be up to 146,000 CF patients across South Asia.[1] There is no access to any of Vertex's CFTR modulators in India or the region. Despite moving to secure multiple patents on your products in India, Vertex has not engaged with the health system or government to advance talks that might result in patients there being treated.

A coalition of CF patients and their families have written to the government seeking the revocation of Vertex's patents under Sections 66 and 100 of the 1970 Patent Act. The revocation of the patent is justified on at least two counts, including Vertex's failure to work their patents (i.e. supply the relevant medicine) in the Indian market, and their unaffordable high prices.

You must now meaningfully engage with the Indian health system in a way that will enable access, drop your patent claims and either drop your prices to genuinely affordable levels for patients in India or facilitate generic supply by applying for registration.

[1] https://www.sciencedirect.com/science/article/abs/pii/S1098360022008188

Ukraine
There is a diagnosed CF patient population of almost 900 people in Ukraine, but there is also a significant undiagnosed population. According to statistics, the average life expectancy of patients with cystic fibrosis (without systematic treatment) is 17-18 years. There is no access to any of your CFTR modulators, leaving CF patients in Ukraine without any effective treatment.

Despite patenting your products in Ukraine, and thus preventing other companies from importing or locally producing generic versions of its medicines, you have shown you have no intention of entering the Ukrainian market. The Ukrainian Ministry of Health wrote to you twice (22/10/2021 and 31/10/2021) asking you to submit registration documents for Kaftrio/Trikafta but you have still not responded. Many Ukrainian CF patients have died during and since that time.

A community-led effort signed by patients, families, and Ukrainian and international organisations requesting a compulsory licence or an IP waiver on your modulators was sent to the government of Ukraine, demanding action.

In recent press publications, you have stated that you are piloting a drug donation programme - and we understand one of those places is Lviv in Ukraine, but, despite our request for further information, you have failed to supply more details meaning that patients are unaware and therefore unable to access your drugs. This is unacceptable, cruel and deliberately vague. Ethically, you must make the details public so that medicine distribution is open and transparent.

Small pilot donation schemes are, of course, no substitute for equitable access for all CF patients. In order to achieve this you must waive your patent rights in Ukraine, submit registration documents there and drop your price to a genuinely affordable level or help to facilitate a generic supply through the issuance of voluntary licences.

Global CF Access
These four countries are just a fraction of the many, many places where CF patients are dying because of your decisions. You should expect patients around the globe to continue to push for legal intervention and flexibilities to gain access to the medicines they desperately need right now. Alternatively you could help these families and end their suffering by creating sustainable and equitable pathways to access in every country by taking the following steps:

- Immediately commit to dropping all patent claims on CFTR drugs in low and middle-income countries
- Facilitate generic supply by filing for registration across the world
- Agree reimbursement deals in all high-income countries within six months, facilitating generic access where any agreement has not been struck
- Safeguard the health and livelihoods of CF patients by dropping prices in all markets and immediately reversing the reduction in US co-pay support by committing to cover all out-of-pocket expenses of American patients.

We now ask that you personally agree to a virtual meeting with some of our representatives to discuss global access to CFTR modulators as a matter of urgency - lives are being lost every day and patients simply cannot afford to wait.

You and Vertex say ‘patients are at the centre of everything we do’ so now is an opportunity to demonstrate that by giving us your personal time and attention, as you do for investors at your quarterly earnings calls. We would like to have an open and honest discussion around the options that are available – we know that other pharmaceutical companies have used these options to save lives and there is no plausible reason why you and Vertex cannot do the same.

We look forward to hearing from you with potential meeting dates.

Regards,

[Signed by CF representatives and advocates from around the world.]